Opinion

Lisa Jarvis

Congress Needs to Fix the FDA's 'Accelerated' Drug-Approval Process

The agency needs the authority to demand prompt clinical trials to demonstrate that medicines actually work.

The FDA needs to see that all drugs help patients. *Photographer: Stan Honda/AFP via Getty Images*

By Lisa Jarvis
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Congress is targeting a strategy that drug companies have increasingly used to speed up the approval of their medicines. Legislation in the House of Representatives would put more guardrails on the Food

and Drug Administration's so-called accelerated approval pathway.

The bill, from Representative Frank Pallone, a New Jersey Democrat, offers needed fixes for a system that can allow questionably effective – and often breathtakingly expensive – drugs to linger on the market for years.

Understanding this situation requires a bit of background. The FDA devised accelerated approval in 1992 in response to the AIDS crisis. Scientists at the time discovered that certain immune cell levels in patients were an excellent proxy for measuring HIV drugs' efficacy. And the agency saw this as a way to give patients quicker access to promising new drugs that appear to affect the course of a disease but have not yet been shown to treat or cure that disease. In 2012, Congress authorized broader use of "accelerated approval" as part of the FDA Safety and Innovation Act.

Pharmaceutical companies have been allowed to seek approval for their drugs based on evidence of "surrogate outcomes" – that is, changes that predict, rather than directly demonstrate, a clinical effect.

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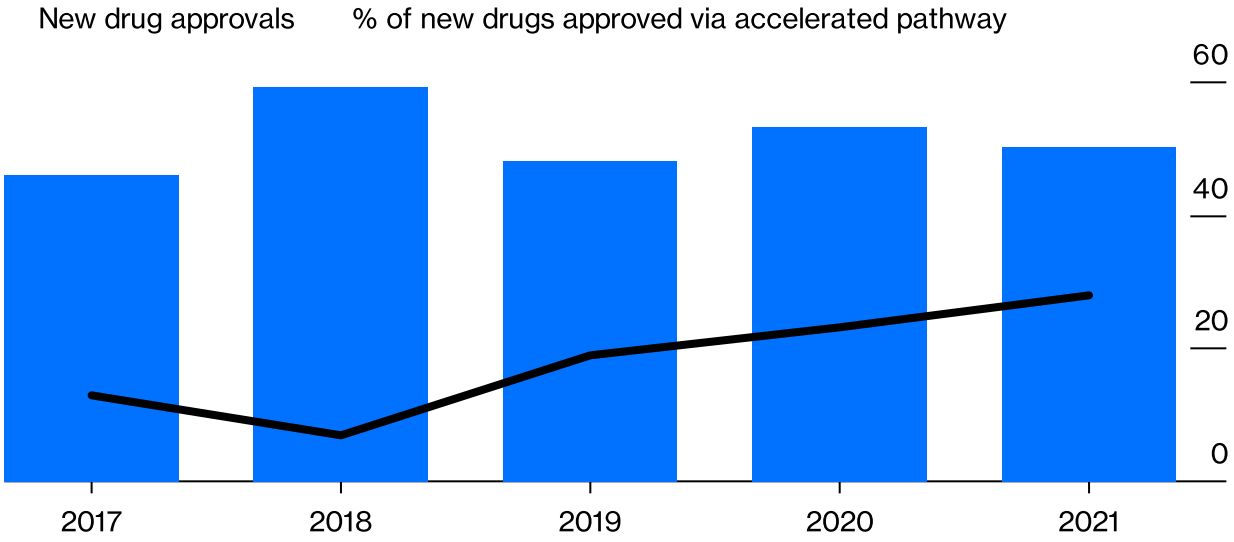
A cancer drug, for example, could be shown to shrink tumors rather than to prolong patient survival. A medicine designed to treat neurodegenerative disease might be shown to increase levels of a critical protein rather than to improve a patient's gait. The assumption is that, with further study, the demonstrated effects will prove to prolong survival or improve mobility – or in some way significantly benefit the patient.

The accelerated approval pathway has helped speed important new drugs to the market. And in the past five years, an increasing share of new drug approvals has relied on it. Last year, more than one in four new medicines – 28% – were given a green light based on surrogate outcomes.

Accelerated Approvals on the Rise

More new drugs are using a regulatory pathway that relies on proxy markers to indicate a clinical benefit



Source: FDA

But the process needs critical tweaks to ensure that studies are promptly done to show that those surrogate outcomes translate into real benefit for patients, without risk of dangerous side effects.

The FDA requires drugmakers to conduct such studies. However, companies have been slow to comply. Products remain on the market for many years – usually at great cost to patients and the health-care system – without strong evidence that they work.

Pallone's legislation would give the FDA greater power to hold drugmakers accountable for conducting the studies without delay.

The need for this fix became obvious last year, after Biogen was granted accelerated approval for its new Alzheimer's treatment, Aduhelm. Clinical trials of this drug had failed to show that it worked to slow cognitive decline. But the FDA approved it anyway – against the recommendation of its own committee of experts – based on a surrogate endpoint, its ability to clear away amyloid plaques from patients' brains.

The FDA gave Biogen an eye-popping nine years to complete a study (not unlike the ones the company had already run without good results) to show that amyloid reduction slows the course of the disease. And the company prepared to sell its new treatment for $56,000 a year. (After an uproar, it cut that price in half.)

Congress took note.

The proposed legislation would not have kept Aduhelm off the market. But it would make it harder for Biogen and other drugmakers to put off the hard work of proving their medicines actually work.

Pallone's bill would require drugmakers to start confirmatory trials as soon as the accelerated approval is granted, not years down the road. That means mapping out the study ahead of the approval. This is an entirely reasonable requirement, and it would allow companies to take advantage of existing clinical trial sites to start enrolling patients. Pallone wants to also require that the trial prove a clinical benefit, not merely rely on the same surrogate markers – a problem commonly seen in post-approval studies of cancer drugs.

Most important, pharma companies would face real consequences for not completing the post-approval studies. If they failed to confirm the drug's benefit within a year of the agreed-upon date (which would be no more than five years out), the approval would expire.

The legislation would also make it easier to take those drugs off the market. The FDA's existing process is clunky and time-consuming. And it's typically used only when a drug fails a post-approval study, not because the study wasn't completed.

While waiting for Congress to provide greater authority, the FDA could also tighten the system somewhat on its own by being more selective about which drugs are allowed to use the accelerated approval pathway. In too many cases, surrogate endpoints take almost as long to measure as good old-fashioned efficacy; the FDA should allow their use only when they offer a meaningful shortcut to the market.

Congress should support Pallone's bill to give the FDA the muscle to ensure that all drugs on the U.S. market are safe and effective.

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